28 February 2019
EMA/CHMP/130311/2019
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Waylivra

volanesorsen

On 28 February 2019, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of conditional marketing authorisation[2] for the medicinal product Waylivra, intended for the treatment of familial chylomicronaemia syndrome (FCS). Waylivra was designated as an orphan medicinal product on 19 February 2014. The applicant for this medicinal product is Akcea Therapeutics Ireland Ltd.

Waylivra will be available as a 285 mg solution for injection. The active substance of Waylivra is volanesorsen, an antisense oligonucleotide which inhibits the formation of apolipoprotein C-III (apoC-III), a protein that regulates both triglyceride metabolism and hepatic clearance of chylomicrons and other triglyceride-rich lipoproteins. Inhibition of apoC-III increases clearance of triglycerides.

The benefits with Waylivra are its ability to reduce levels of fasting triglycerides. The most common side effects are reduced platelet counts and injection site reactions.

The full indication is: “an adjunct to diet in adult patients with genetically confirmed familial chylomicronaemia syndrome (FCS) and at high risk for pancreatitis, in whom response to diet and triglyceride lowering therapy has been inadequate.” It is proposed that Waylivra treatment should be initiated by and remain under the supervision of a physician experienced in the treatment of patients with FCS.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

[2] A conditional marketing authorisation is granted to a medicinal product that fulfils an unmet medical need when the benefit to public health of immediate availability outweighs the risk inherent in the fact that additional data are still required. The marketing authorisation holder is likely to provide comprehensive clinical data at a later stage.

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